Multiple Sclerosis therapy: Human Pluripotent Stem Cell-Derived Neural Progenitor Cells

Grant Award Details

Multiple Sclerosis therapy: Human Pluripotent Stem Cell-Derived Neural Progenitor Cells

Grant Type: Early Translational III

Grant Number: TR3-05603

Project Objective: To provide proof-of-concept for a human pluripotent stem cell (hPSC)-based development candidate (DC) for the treatment of
Multiple Sclerosis (MS). The strategy is to bank iPSC and ES lines with common HLA haplotypes that will be used to generate enriched populations of a specific type of human neural precursor cells (hNPCs), perform preclinical testing in two well-accepted animal models of multiple sclerosis and identify he best hNPC lines to bring forward to human studies.

Investigator:

Name:	Craig Walsh
Institution:	University of California, Irvine
Type:	PI

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Name:	Jeanne Loring
Institution:	Scripps Research Institute
Type:	Co-PI

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Name:	Claude Bernard
Institution:	Monash University
Type:	Partner-PI

Disease Focus: Multiple Sclerosis, Neurological Disorders

Collaborative Funder: Australia

Human Stem Cell Use: Embryonic Stem Cell, iPS Cell

Award Value: $4,535,005

Status: Closed

Progress Reports

Reporting Period: Year 1

View Report

Reporting Period: Year 2

View Report

Reporting Period: Year 3 + NCE

View Report

Grant Application Details

Application Title: Multiple Sclerosis therapy: Human Pluripotent Stem Cell-Derived Neural Progenitor Cells

Public Abstract: Multiple Sclerosis (MS) is a disease of the central nervous system (CNS) caused by inflammation and loss of cells that produce myelin, which normally insulates and protects nerve cells. MS is a leading cause of neurological disability among young adults in North America. Current treatments for MS include drugs such as interferons and corticosteroids that modulate the ability of immune system cells to invade the CNS. These therapies often have unsatisfactory outcomes, with continued progression of neurologic disability over time. This is most likely due to irreversible tissue injury resulting from permanent loss of myelin and nerve destruction. The limited ability of the body to repair damaged nerve tissue highlights a critically important and unmet need for MS patients. The long-term goal of our research is to develop a stem cell-based therapy that will not only halt ongoing loss of myelin but also lead to remyelination and repair of damaged nerve tissue. Our preliminary data in animal models of human MS are very promising and suggest that this goal is possible. Research efforts will concentrate on refining techniques for production and rigorous quality control of clinically-compatible transplantable cells generated from high-quality human pluripotent stem cell lines, and to verify the therapeutic activity of these cells. We will emphasize safety and development of the most therapeutically beneficial cell type for eventual use in patients with MS.

Statement of Benefit to California:	One in seven Americans lives in California, and these people make up the single largest health care market in the United States. The diseases and injuries that affect Californians affect the rest of the US and the world. Many of these diseases involve degeneration of healthy cells and tissues, including neuronal tissue in diseases such as Multiple Sclerosis (MS). The best estimates indicate that there are 400,000 people diagnosed with MS in the USA and 2.2 million worldwide. In California, there are approximately 160,000 people with MS – roughly half of MS patients in the US live in California. MS is a life-long, chronic disease diagnosed primarily in young adults who have a virtually normal life expectancy but suffer from progressive loss of motor and cognitive function. Consequently, the economic, social and medical costs associated with the disease are significant. Estimates place the annual cost of MS in the United States in the billions of dollars. The development of a stem cell therapy for treatment of MS patients will not only alleviate ongoing suffering but also allow people afflicted with this disease to return to work and contribute to the economic stabilization of California. Moreover, a stem cell-based therapy that will provide sustained recovery will reduce recurrence and the ever-growing cost burden to the California medical community.
